



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2005-D-0460]

Pediatric Drug Development Under the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act: Scientific Considerations; Draft Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of availability.

SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of a draft guidance for industry entitled “Pediatric Drug Development Under the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act: Scientific Considerations.” This draft guidance addresses selected clinical, scientific, and ethical issues involved in developing drugs, including biological products, for pediatric use when such drug products are subject to the Pediatric Research Equity Act (PREA) and/or the Best Pharmaceuticals for Children Act (BPCA). This draft guidance is intended to assist industry in obtaining the data and information necessary to support the approval of drug products in pediatric populations. This draft guidance does not address the clinical development of drug products that are not subject to either PREA or the BPCA.

DATES: Submit either electronic or written comments on the draft guidance by [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*] to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.

ADDRESSES: You may submit comments on any guidance at any time as follows:

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: <https://www.regulations.gov>. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to <https://www.regulations.gov> will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on <https://www.regulations.gov>.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in “Instructions.”

Instructions: All submissions received must include the Docket No. FDA-2005-D-0460 for “Pediatric Drug Development Under the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act: Scientific Considerations.” Received comments will be placed in the docket and, except for those submitted as “Confidential Submissions,” publicly viewable at <https://www.regulations.gov> or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday, 240-402-7500.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states “THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION.” The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on <https://www.regulations.gov>. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as “confidential.” Any information marked as “confidential” will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA’s posting of comments to

public docket, see 80 FR 56469, September 18, 2015, or access the information at:

<https://www.govinfo.gov/content/pkg/FR-2015-09-18/pdf/2015-23389.pdf>.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to <https://www.regulations.gov> and insert the docket number, found in brackets in the heading of this document, into the “Search” box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852, 240-402-7500.

You may submit comments on any guidance at any time (see 21 CFR 10.115(g)(5)).

Submit written requests for single copies of the draft guidance to the Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001 New Hampshire Ave., Hillandale Bldg., 4th Floor, Silver Spring, MD 20993-0002, or the Office of Communication, Outreach and Development, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 3128, Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist that office in processing your requests. See the **SUPPLEMENTARY INFORMATION** section for electronic access to the draft guidance document.

FOR FURTHER INFORMATION CONTACT: Rosemary Addy, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, Rm. 6430, Silver Spring, MD 20993-0002, 301-796-2200, pedsdrugs@fda.hhs.gov; or Diane Maloney, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled “Pediatric Drug Development Under the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act: Scientific Considerations.” This draft guidance addresses selected clinical, scientific, and ethical issues involved in developing drugs for pediatric use when such drugs are subject to PREA and/or the BPCA. This draft guidance, along with the draft guidance entitled “Pediatric Drug Development: Regulatory Considerations--Complying With the Pediatric Research Equity Act and Qualifying for Pediatric Exclusivity Under the Best Pharmaceuticals for Children Act,” revises and replaces the draft guidance entitled “How to Comply with the Pediatric Research Equity Act.”¹ This draft guidance also addresses certain additional topics that FDA has not previously addressed in guidance.

The purpose of this draft guidance is to assist industry in obtaining the data and information needed to support approval of drug products in pediatric populations. Specifically, this draft guidance describes considerations regarding data in pediatric patients with particular discussion regarding formulation development, nonclinical information, clinical pharmacology, and safety information. Additionally, the draft guidance discusses pediatric extrapolation, timing of pediatric studies, and drug development for the neonatal population. This draft guidance does not address the clinical development of drugs that are not subject to either PREA or the BPCA.

¹ This draft guidance also addresses certain topics previously addressed in the guidance for industry entitled “Qualifying for Pediatric Exclusivity Under Section 505A of the Federal Food, Drug, and Cosmetic Act.” That guidance was withdrawn August 7, 2013 (78 FR 48175).

This draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent the current thinking of FDA on "Pediatric Drug Development Under the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act: Scientific Considerations." It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations.

II. The Paperwork Reduction Act of 1995

While this guidance contains no collection of information, it does refer to previously approved FDA collections of information. Therefore, clearance by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (PRA) (44 U.S.C. 3501-3521) is not required for this guidance. The previously approved collections of information are subject to review by OMB under the PRA. The collections of information in 21 CFR parts 50 and 56 for protection of human subjects and institutional review boards, have been approved under OMB control number 0910-0130. The collections of information in 21 CFR 210 and 211 for current good manufacturing practice have been approved under OMB control number 0910-0139. The collections of information in 21 CFR part 312 for investigational new drug applications and 21 CFR part 314 for new drug applications and abbreviated new drug applications have been approved under OMB control numbers 0910-0014 and 0910-0001, respectively. The collections of information in 21 CFR parts 601 and 610 for biologics license applications have been approved under OMB control number 0910-0338. The collections of information in 42 U.S.C. 262(k) and 21 U.S.C. 379g for biosimilar applications have been approved under OMB control number 0910-0718. The collections of information in 21 CFR 201.56 and 201.57 regarding labeling requirements for prescription drugs have been approved under OMB control number 0910-0572. The collections of information in 21 CFR part 201, subpart C regarding over-the-

counter products have been approved under OMB control number 0910-0340. The collections of information in 21 CFR part 316 regarding orphan drug product development are approved under OMB control number 0910-0167.

III. Electronic Access

Persons with access to the internet may obtain the draft guidance at <https://www.fda.gov/drugs/guidance-compliance-regulatory-information/guidances-drugs>, <https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances>, <https://www.fda.gov/regulatory-information/search-fda-guidance-documents>, or <https://www.regulations.gov>.

Dated: May 15, 2023.

Lauren K. Roth,

Associate Commissioner for Policy.

[FR Doc. 2023-10611 Filed: 5/17/2023 8:45 am; Publication Date: 5/18/2023]